Use of PROMs in routine clinical practice:
The experience of the PH Paris Center

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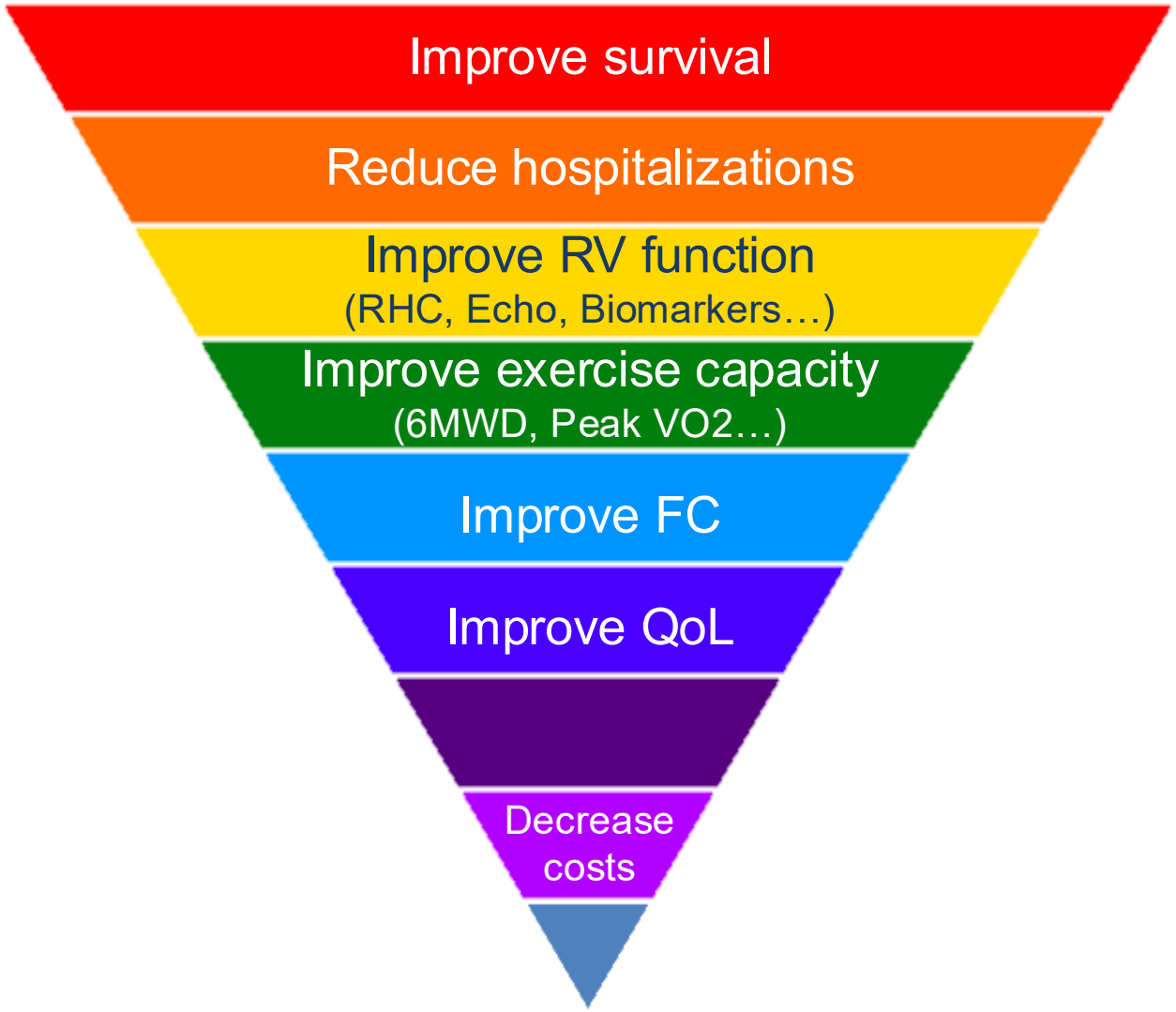
Conflict of interest disclosure

❑ I have the following real or perceived conflicts of interest that relate to this presentation:

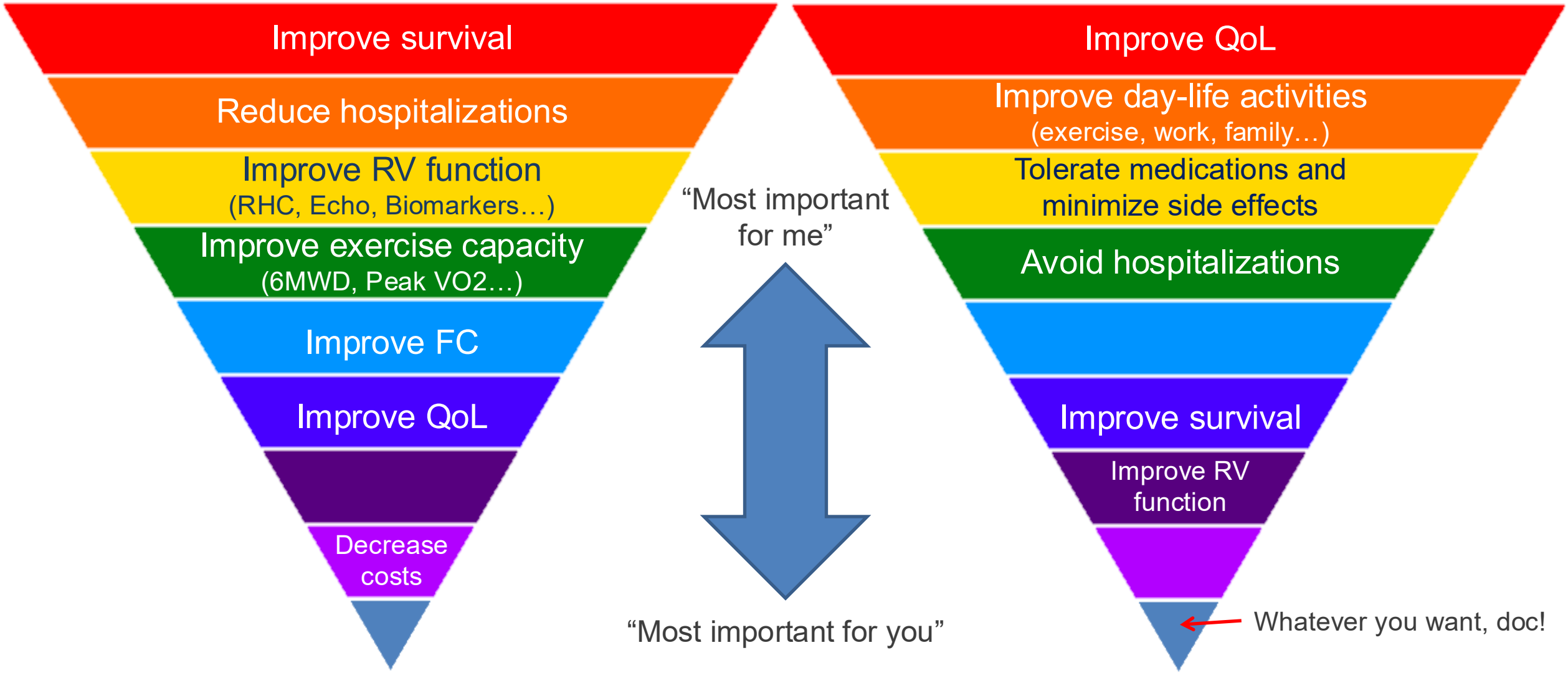
Affiliation / Financial interest	Commercial company
Grants/research support:	AOP Orphan, Ferrer, Gossamer Bio, Janssen, MSD
Honoraria or consultation fees:	Aerovate, AOP Orphan, Enzyvant, Ferrer, Gossamer Bio, Janssen, Liquidia, MSD, Pulmovant, Respira Therapeutics, United Therapeutics,
Participation in a company sponsored bureau:	No
Stock shareholder:	No
Spouse / partner:	No
Other support / potential conflict of interest:	No

Treatment goal in PAH: A physician's perspective…



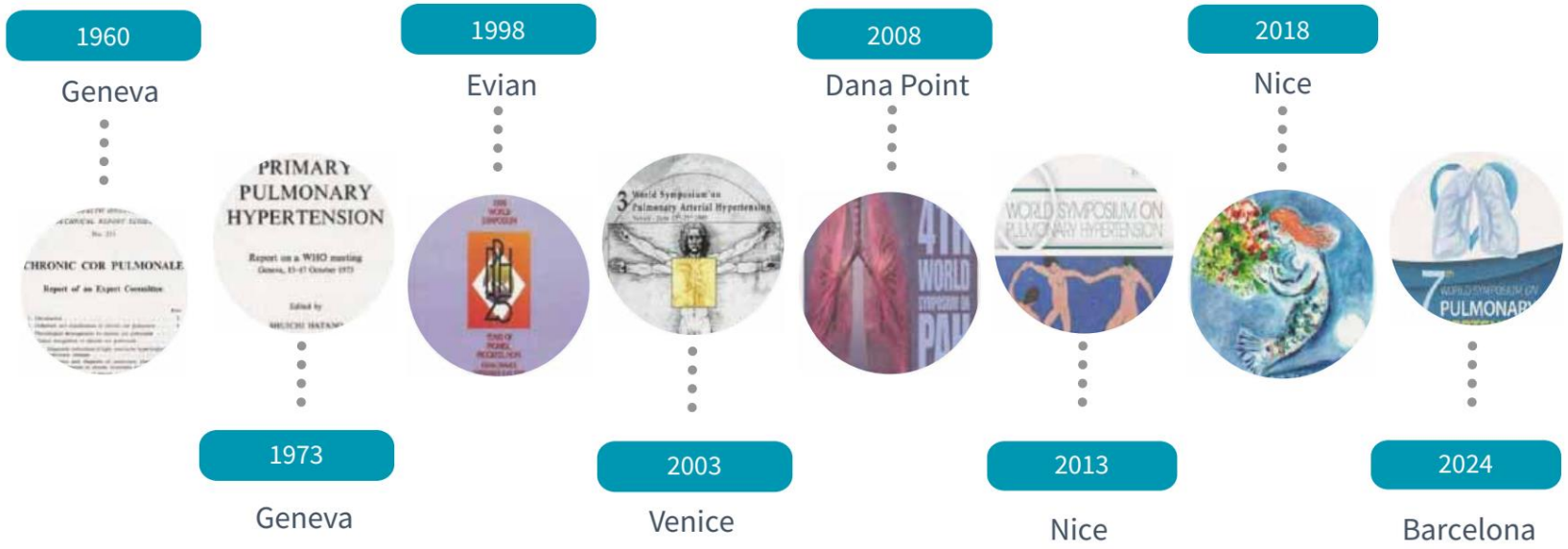
Adapted from a slide courtesy of Jean-Luc Vachiery, and Zannad F. *et al. Eur J Heart Fail* 2013; 15: 1082–94.

What is relevant to patients is very different!



Adapted from a slide courtesy of Jean-Luc Vachiery, and Zannad F. *et al. Eur J Heart Fail* 2013; 15: 1082–94.

Patient perspective: task force #1 at the 7th WSPH



EUROPEAN RESPIRATORY JOURNAL
TASK FORCE REPORT
H.J. FORD ET AL.

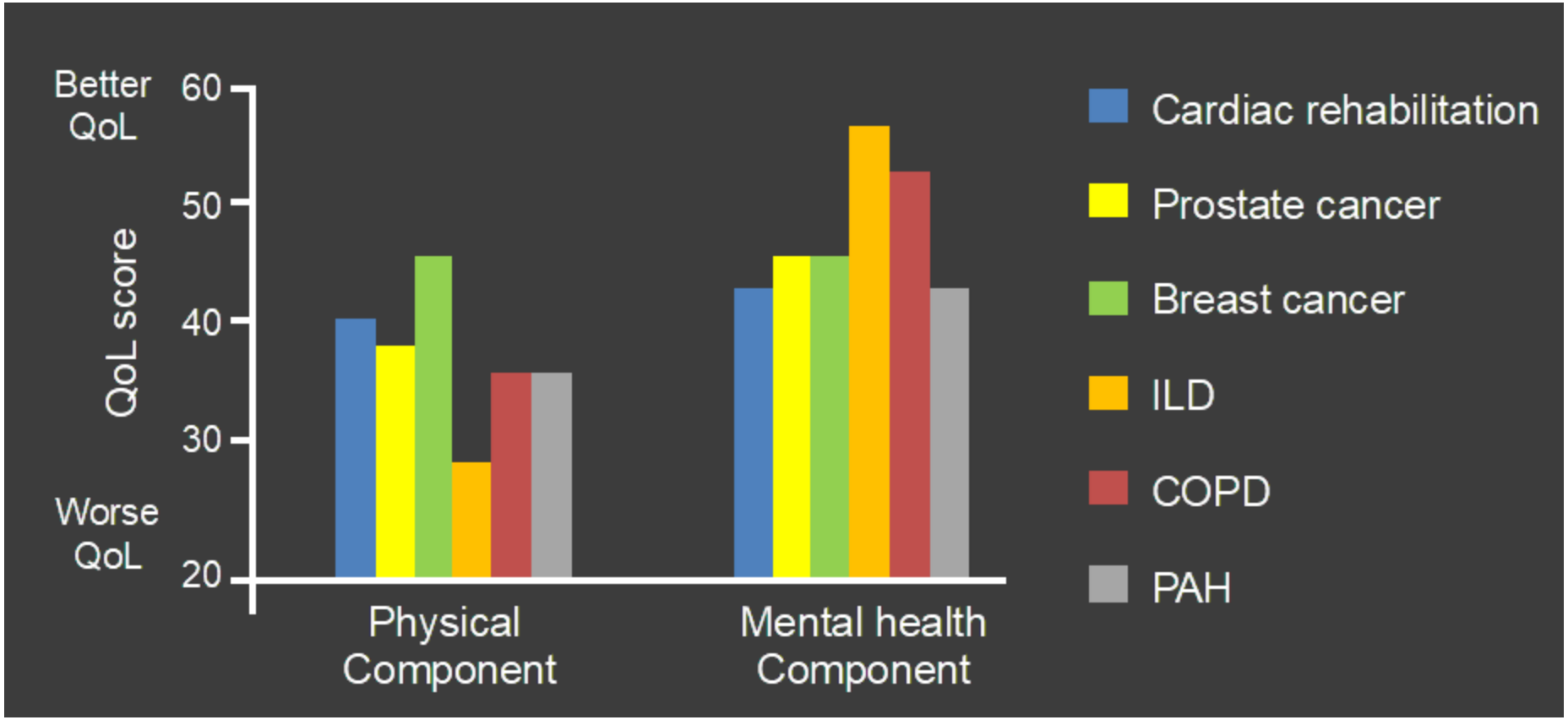
Exploring the patient perspective in pulmonary hypertension

H. James Ford [1], Colleen Brunetti [2], Pisana Ferrari [3], Gergely Meszaros [4], Victor M. Moles [5], Hall Skaara [6], Adam Torbicki [7] and J. Simon R. Gibbs [8]

Humbert M, *et al. Eur Respir J* 2024; 64: 2401222; Ford HJ, *et al. Eur Respir J* 2024; 64: 2401129.

The effect of PAH on health-related quality of life (HRQoL) is as severe as in other serious debilitating conditions

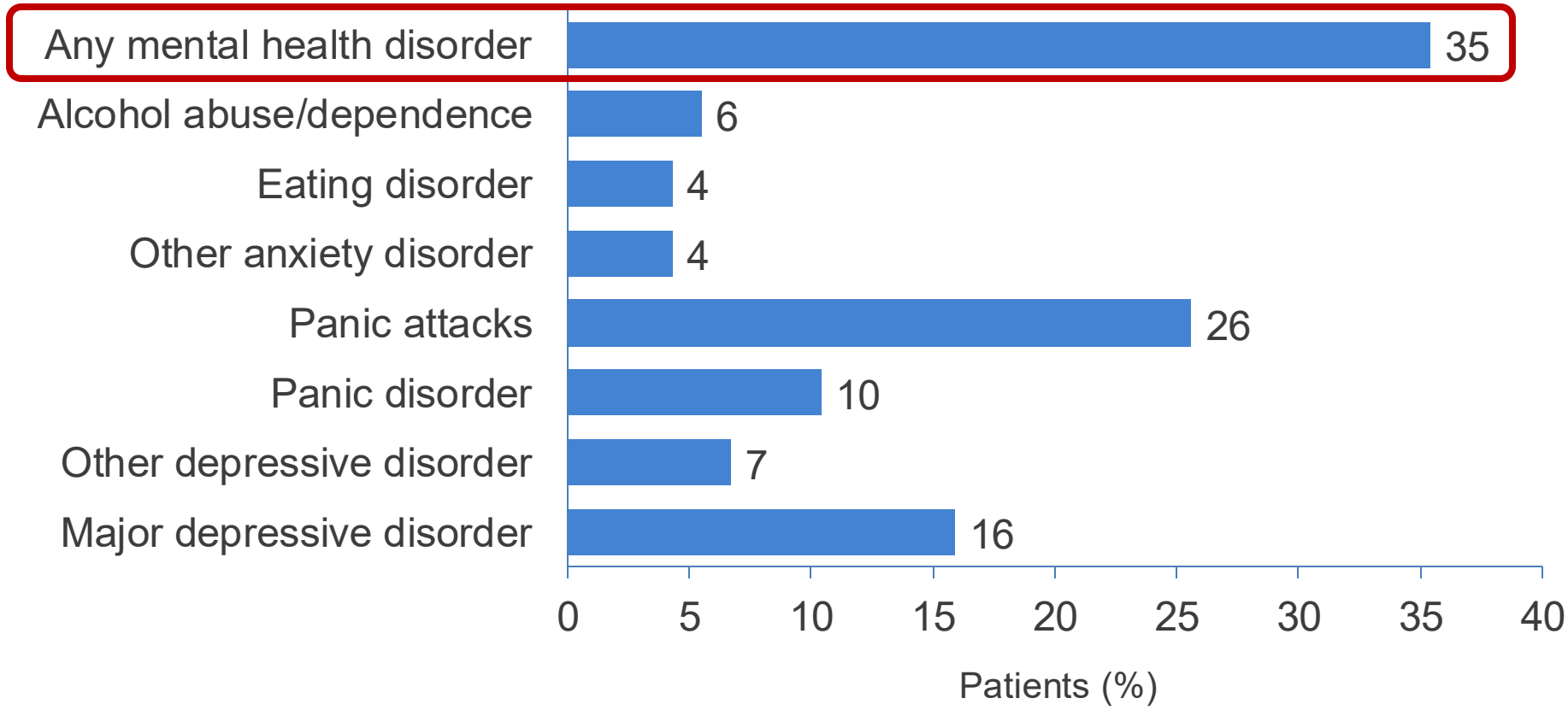
Impact of different chronic diseases on HRQoL (SF-36)



Mean scores for the physical and mental component summary scores of the SF-36

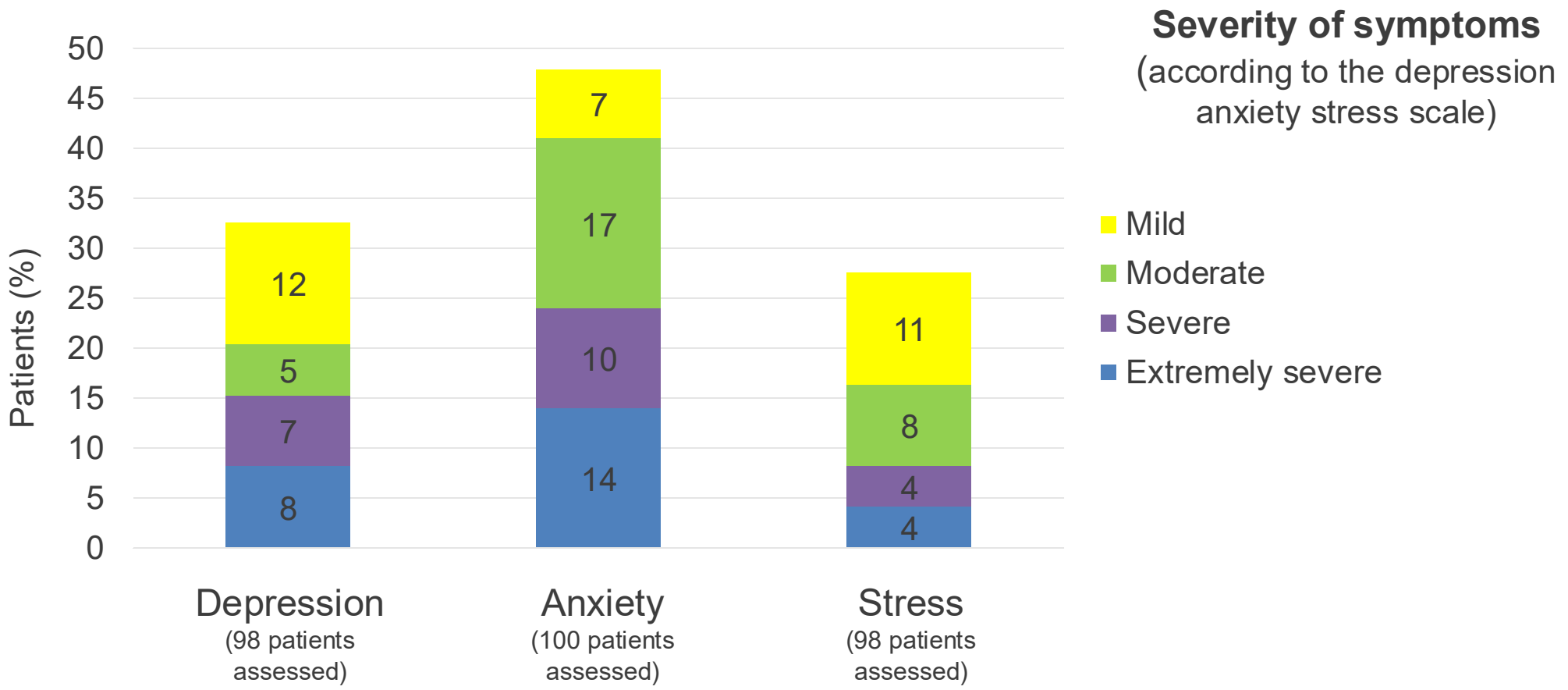
Taichman DB, *et al. Respir Res* 2005; 6:92.

More than a third of PH patients experience mental disorders



Lowe B, *et al*. *Psychosom Med* 2004; 66: 831-36.

PAH patients suffer from depression, anxiety and stress



Vanhoof JMM, *et al*. *J Heart Lung Transplant* 2014; 33:800-8.

Assessment of HRQoL

- Broad, complex and subjective[1]
- Individual physical and mental health perceptions[1]
  - Health risks and conditions
  - Functional status[2]
  - Social support[3]
  - Socioeconomic status[4]
- From “How are you doing today?” and functional class assessment

…to HRQoL / Patient-Reported Outcomes Measures (PROM)

1. Howard L, *et al. Eur Resp Rev* 2014; 23:458-68; 2. Taichman DB, *et al. Respir Res* 2005; 6:92;
3. Pragodpol P and Ryan C. *J Cardiovasc Nurs* 2013; 28:277-84; 4. Mielck A, *et al. Health Qual Life Outcomes* 2014; 12:58.

Functional class is difficult to assess…

Factors PH-experienced clinicians* considered when formulating FC assessments

	Paper	Online	*P* value
Symptoms	41 (93)	68 (99)	.13
Medication used	10 (23)	17 (25)	>.99
Hemodynamic values	11 (25)	0	<.001
Physical examination	24 (55)	31 (45)	.34
6-minute walk distance	18 (41)	41 (59)	.08
Echocardiographic findings	8 (18)	0	<.001
Patient's lifestyle	33 (75)	46 (67)	.40
Patient's occupation	23 (52)	31 (45)	.56
Insurance requirements	9 (20)	0	.01

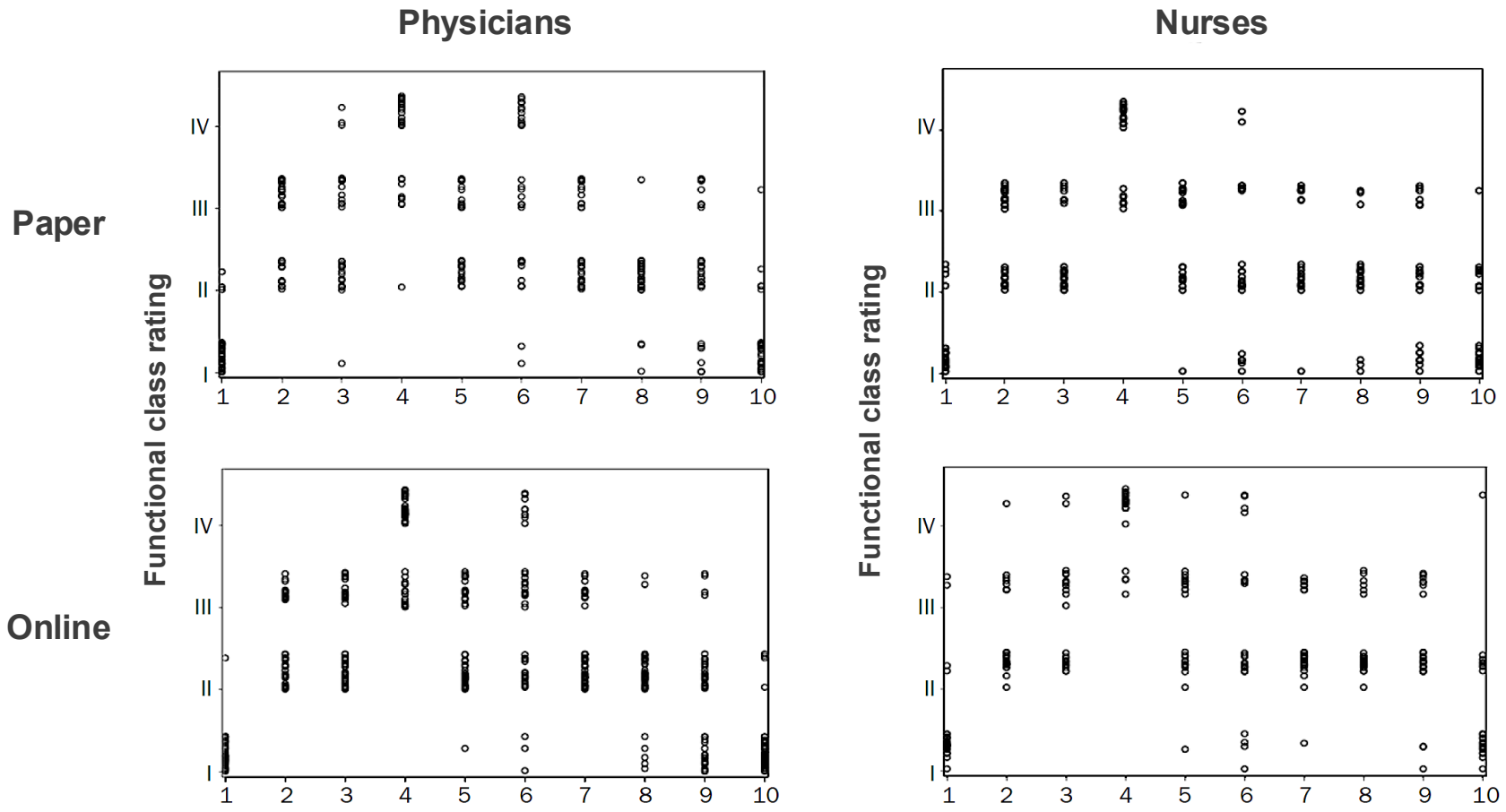
Data are number (percentage).

*10 patient-case scenarios (paper & online) analysed by PH-experienced physicians (n=68) and nurses (n=45)

Taichman DB, *et al*. *Mayo Clin Proc* 2009;84:586-92.

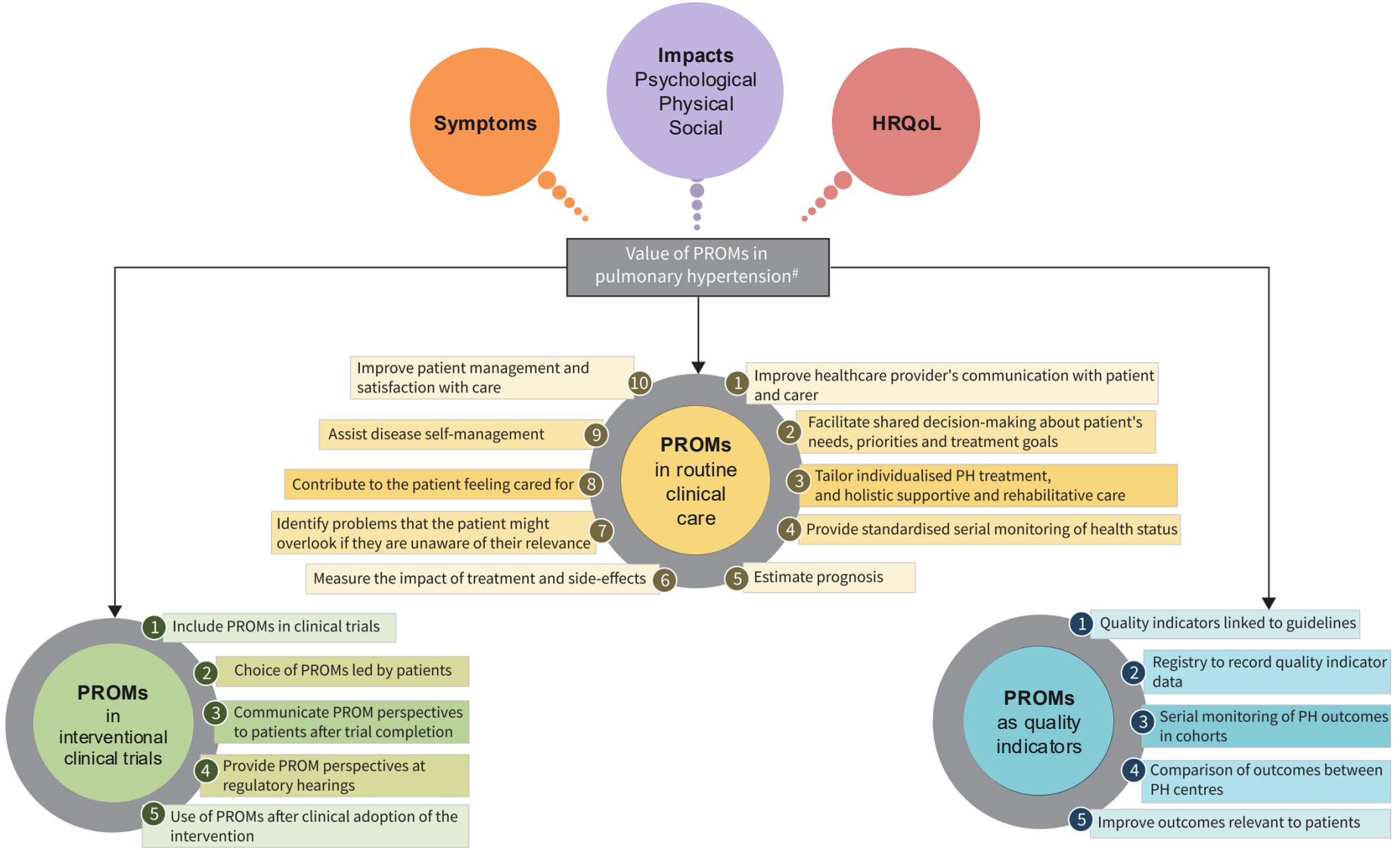
Significant variations in the clinical assessment of FC in patients with PAH

10 patient-case scenarios analysed by PH-experienced physicians and nurses



Taichman DB, *et al*. *Mayo Clin Proc* 2009;84:586-92.

PROMs: potential value and what they measure



Ford HJ, *et al*. *Eur Respir J* 2024; 64: 2401129.

PROMs used in routine clinical care and research for PH

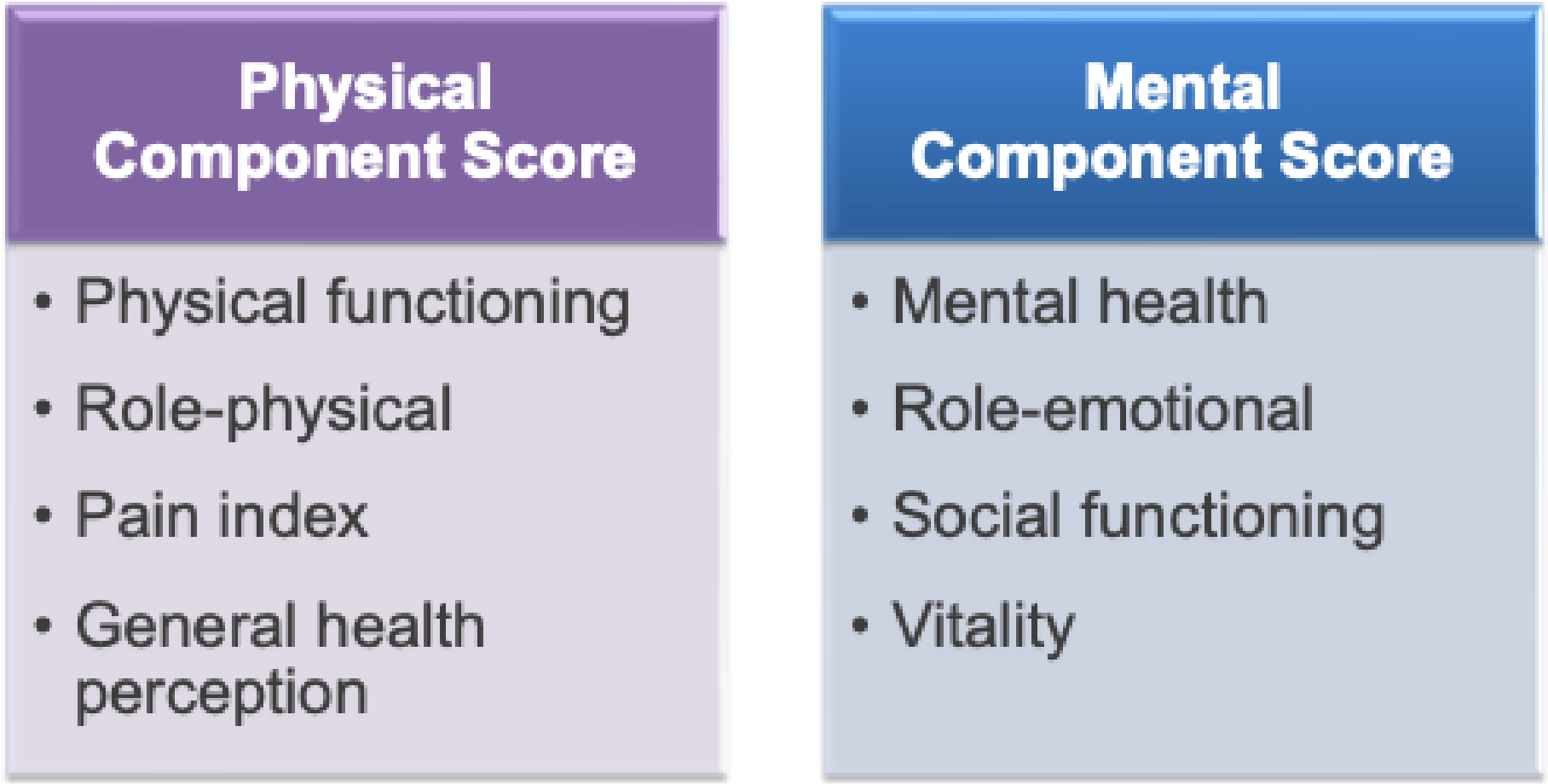
PH-specific

Non-specific

PROM	Diseases assessed	Domains assessed	Correlates with	Scale/ scoring	Questions n	Approximate completion time (min)	Time period assessed	Languages available
CAMPHOR	PAH CTEPH	Symptoms Activity HRQoL	6MWD, FC, Borg dyspnoea score, clinical worsening	Yes/no and 3-point Likert	65	10	Same day	23
EmPHasis-10	PAH CTEPH	HRQoL	6MWD, FC, BNP, PVR, REVEAL risk score, survival	6-point semantic differential	10	2–3	At time of assessment	25
Living with PH	PAH	Physical Emotional	6MWD, FC	6-point Likert	21	5–10	1 week	English only
PAH-SYMPACT	PAH CTEPH	Cardiopulmonary symptoms Cardiovascular symptoms Physical impacts Cognitive/ emotional impacts	6MWD, FC, REVEAL 2.0 risk score D_{LCO}, survival	5-point Likert	23	5–7	24 h for symptoms 7 days for impacts	22 for paper version, 33 for electronic version
PAHSIS	PAH	Symptoms	SF-36 scores	11-point Likert	17	<5	1 month	English only
EQ-5D	Generic	Mobility Self-care Pain Anxiety/depression Activity	Not available for PAH	Visual analogue	5	2–5	Same day	208
SF-36	Generic	Physical functioning Physical limitations Pain General health Energy/vitality Social functioning Emotional limitations Mental health	6MWD, FC, survival	Variable	36	8–10	4 weeks	193

Ford HJ, *et al*. *Eur Respir J* 2024; 64: 2401129.

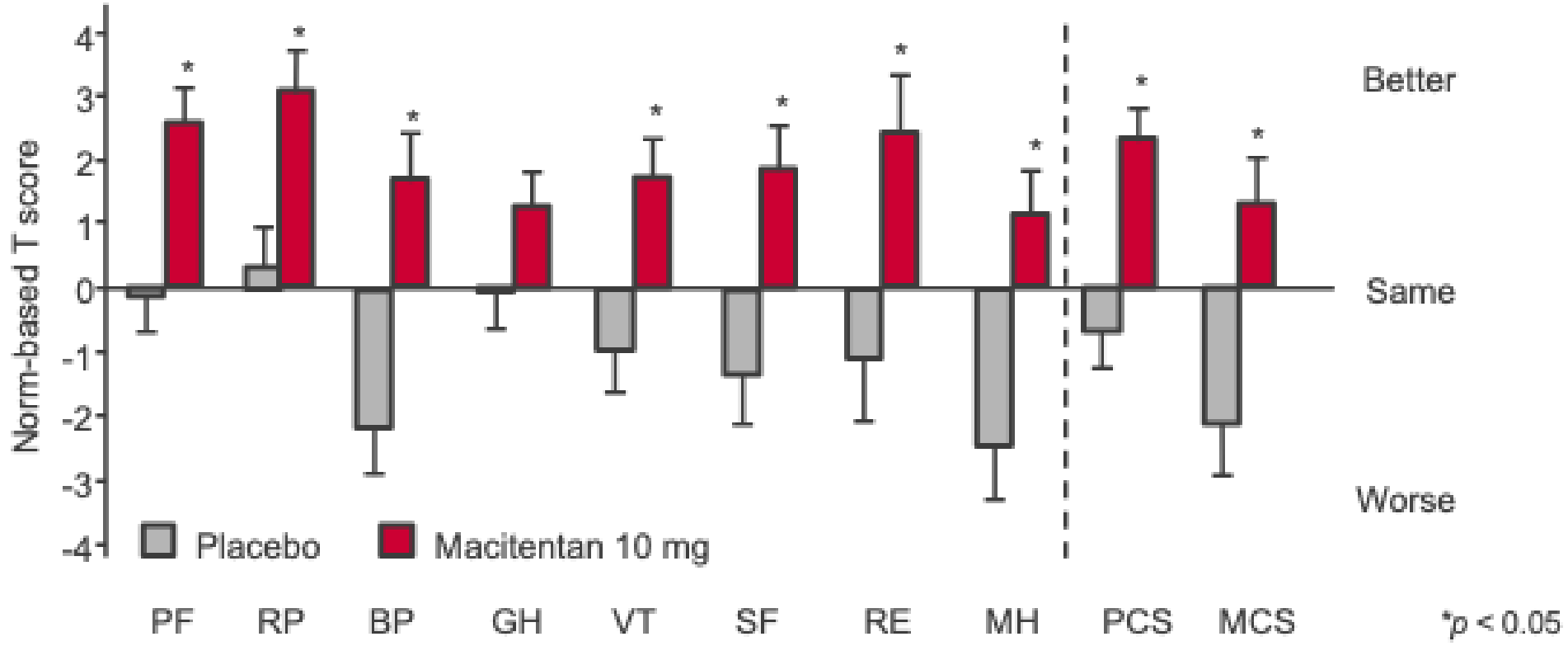
SF-36 questionnaire (non-specific HRQoL assessment)



Ware JE Jr, Sherbourne CD. The MOS 36-Item Short-Form Health Survey (SF-36): I. Conceptual Framework and Item Selection. *Medical Care*. 1992; 30: 473-483.

PAH management improves HRQoL assessed with SF-36

Changes in patient HRQoL from baseline to month 6 assessed by **SF-36 scores in the SERAPHIN study**

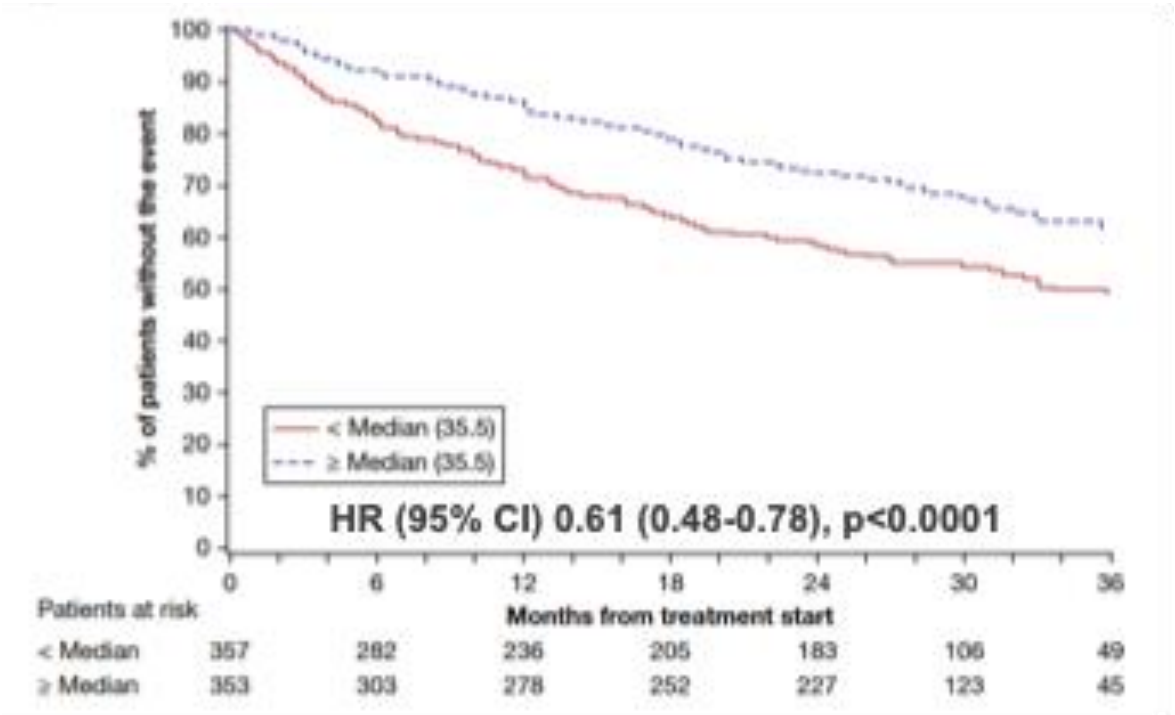


PF: physical functioning; RP: role physical; BP: bodily pain; GH: general health; VT: vitality; SF: social functioning; RE: role emotional; MH: mental health; PCS: physical component score; MCS: mental component score

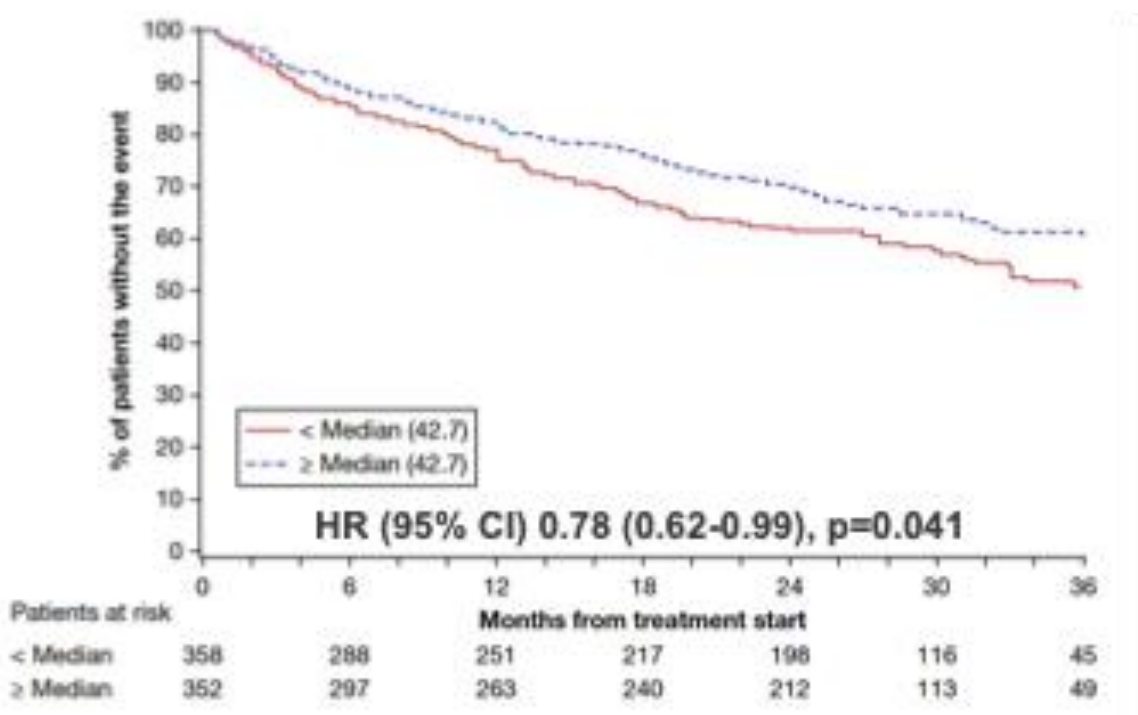
Mehta S, *et al. Chest* 2017;151:106–18.

Baseline SF-36 component score is associated with outcomes

Physical component



Mental component

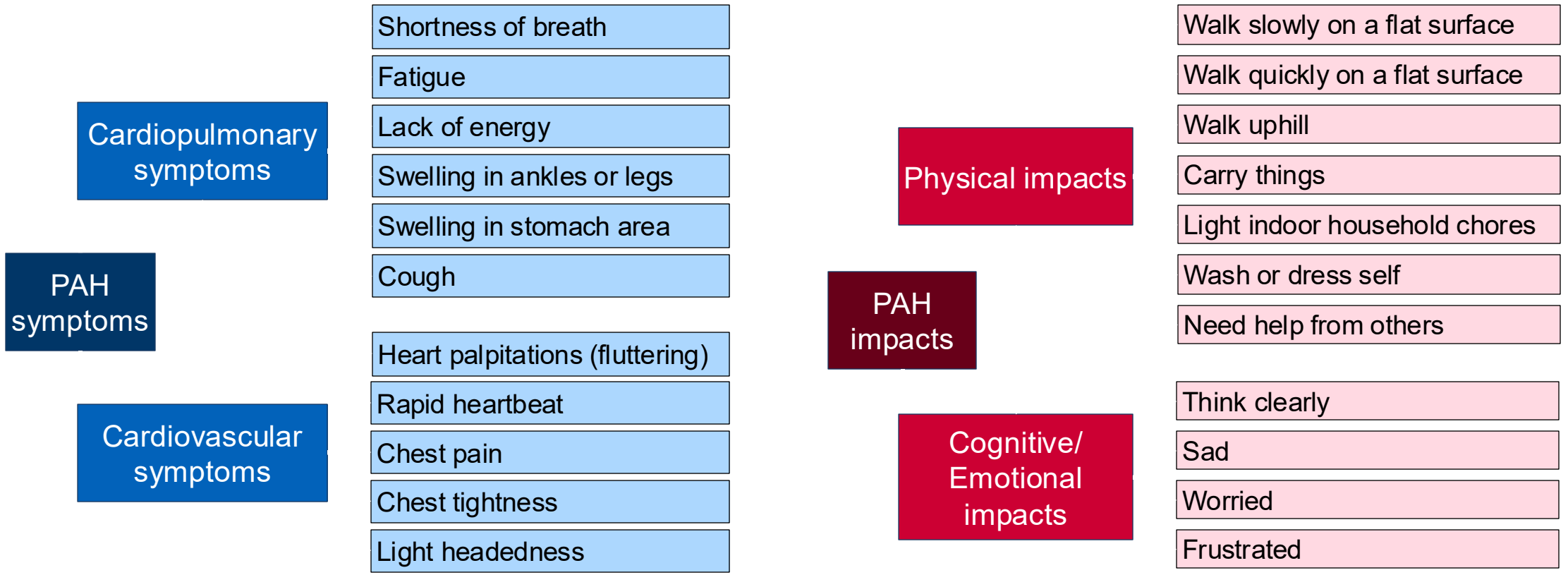


K-M analyses of **time to first confirmed morbidity or mortality event** according to **median baseline SF-36** component summary score in the **SERAPHIN study**

Mehta S, *et al. Chest* 2017;151:106–18.

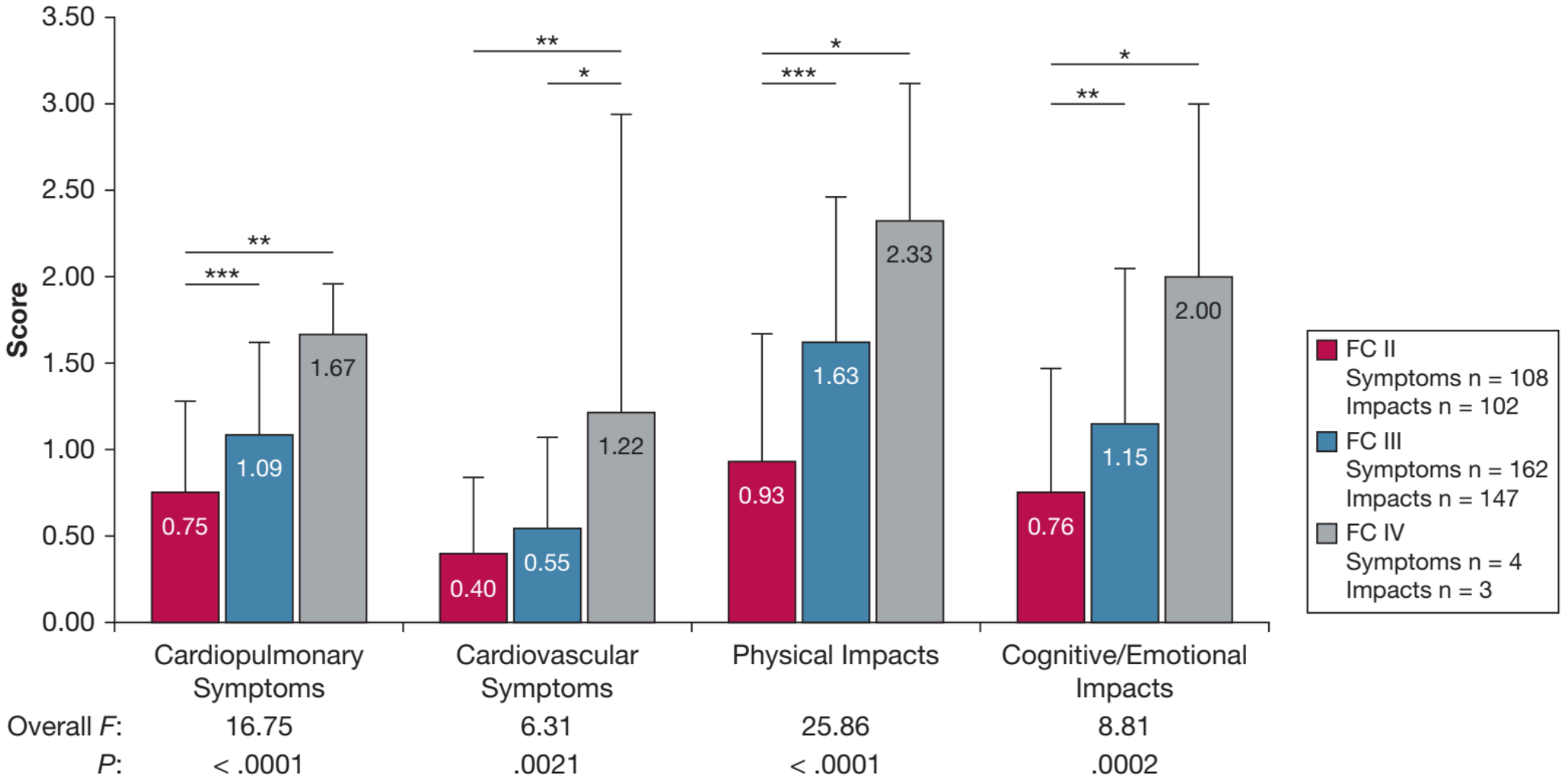
PAH-SYMPACT® conceptual framework

- PAH-specific PRO tool developed in accordance with the FDA PRO guidance
- Evaluation of 11 PAH symptoms and 11 impact items (plus one item on oxygen use)



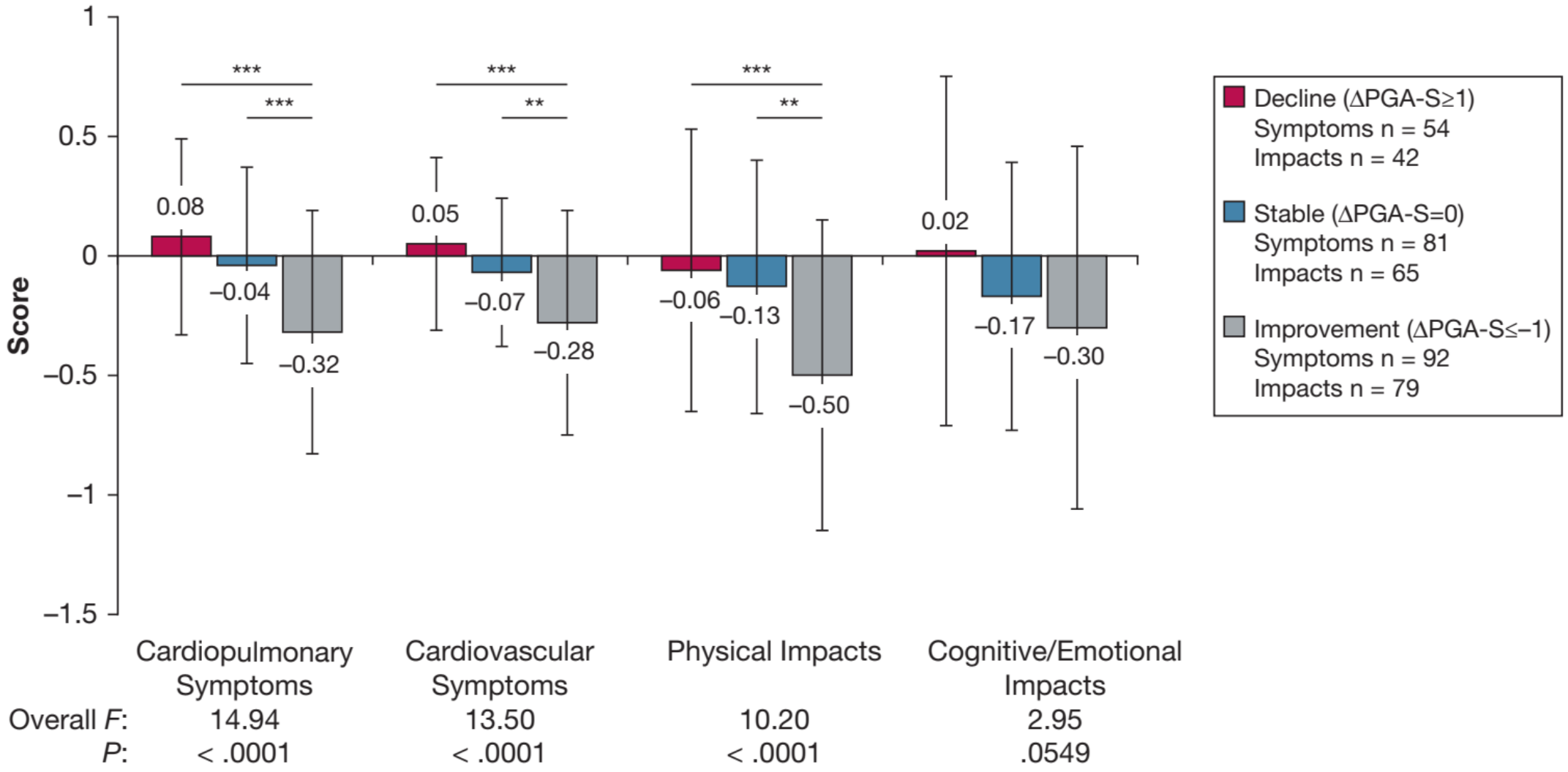
Chin KM, *et al*. *CHEST* 2018; 154: 848-861.

PAH-SYMPACT®: Domain scores according to functional class



Chin KM, *et al*. *CHEST* 2018; 154: 848-861.

PAH-SYMPACT®: Change over time in domain scores



Chin KM, *et al*. *CHEST* 2018; 154: 848-861.

emPHasis-10 questionnaire

- Measures the **impact of PH on HRQoL**
- Developed from interviews conducted with 30 PH patients
- Semantic differential **six-point scale** (0–5)
- Short **self-administered** questionnaire
- **Sensitive to differences** in relevant clinical parameters
- **Freely available** for clinical and academic use
- Translated in **25 languages**

emPHasis10

NHS/Hospital number:

Name:

Date of birth:

This questionnaire is designed to determine how pulmonary hypertension (PH) affects your life. Please answer every question by placing a tick over the ONE NUMBER that best describes your recent experience of living with PH.

For each item below, place a tick (✓) in the box that best describes your experience.

I am not frustrated by my breathlessness	0 1 2 3 4 5	I am very frustrated by my breathlessness
Being breathless never interrupts my conversations	0 1 2 3 4 5	Being breathless always interrupts my conversations
I do not need to rest during the day	0 1 2 3 4 5	I always need to rest during the day
I do not feel exhausted	0 1 2 3 4 5	I always feel exhausted
I have lots of energy	0 1 2 3 4 5	I have no energy at all
When I walk up one flight of stairs I am not breathless	0 1 2 3 4 5	When I walk up one flight of stairs I am very breathless
I am confident out in public places/crowds despite my PH	0 1 2 3 4 5	I am not confident at all in public places/crowds because of my PH
PH does not control my life	0 1 2 3 4 5	PH completely controls my life
I am independent	0 1 2 3 4 5	I am completely dependent
I never feel like a burden	0 1 2 3 4 5	I always feel like a burden

Total:

Date:

pha UK pulmonary hypertension association

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Yorke J, *et al. Eur Respir J* 2014; 43:1106-13.

Use of emPHasis10 in clinical practice in the French PH Network

- French version of emPHasis10 used within the French PH Network since January 2024

- 2283 emPHasis10 scores recorded in 1448 patients
  - 230 incident patients (newly diagnosed)
  - 1218 prevalent patients
  - 461 patients with two or more questionnaires, including 98 with baseline assessment

emPHasis10

Numéro de sécurité sociale :

Nom :

Date de naissance :

Ce questionnaire est destiné à déterminer comment l'hypertension pulmonaire (HP) affecte votre vie. Veuillez répondre à chaque question en cochant LE CHIFFRE qui décrit le mieux votre expérience quotidienne récente en ce qui concerne l'hypertension pulmonaire.

Pour chaque élément ci-dessous, veuillez cocher (✓) la case qui décrit le mieux votre expérience.

Je ne suis pas contrarié(e) par mon essoufflement	0 1 2 3 4 5	Je suis très contrarié(e) par mon essoufflement
Le fait d'être essoufflé(e) n'interrompt jamais mes conversations	0 1 2 3 4 5	Le fait d'être essoufflé(e) interrompt toujours mes conversations
Je n'ai pas besoin de me reposer pendant la journée	0 1 2 3 4 5	J'ai toujours besoin de me reposer pendant la journée
Je ne me sens pas épuisé(e)	0 1 2 3 4 5	Je me sens toujours épuisé(e)
J'ai beaucoup d'énergie	0 1 2 3 4 5	Je n'ai aucune énergie
Je ne suis pas essoufflé(e) lorsque je monte un étage à pied	0 1 2 3 4 5	Je suis très essoufflé(e) lorsque je monte un étage à pied
J'ai confiance en moi lorsque je suis dans un lieu public/au milieu de la foule malgré mon HP	0 1 2 3 4 5	Je n'ai pas confiance en moi lorsque je suis dans un lieu public/au milieu de la foule à cause de mon HP
L'HP ne contrôle pas ma vie	0 1 2 3 4 5	L'HP contrôle totalement ma vie
Je suis indépendant(e)	0 1 2 3 4 5	Je suis totalement dépendant(e)
Je n'ai jamais l'impression d'être un fardeau	0 1 2 3 4 5	J'ai toujours l'impression d'être un fardeau

Total :

Date :

pha UK pulmonary hypertension association

French (France) 06 January 2015
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Boucly A. Data on file.

Patient demographics (n = 1448)

Sex (male / female), n (%)	615 (42) / 833 (58)
Age, years	62 ± 16
Aetiology, n(%) • PAH • Group 2 • Group 3 • CTEPH • Group 5	 807 (56) 16 (1) 204 (14) 326 (22.5) 95 (6.5)
Incidents, n (%)	230 (16)
Death / lung transplantation	44 (3) / 11 (1)

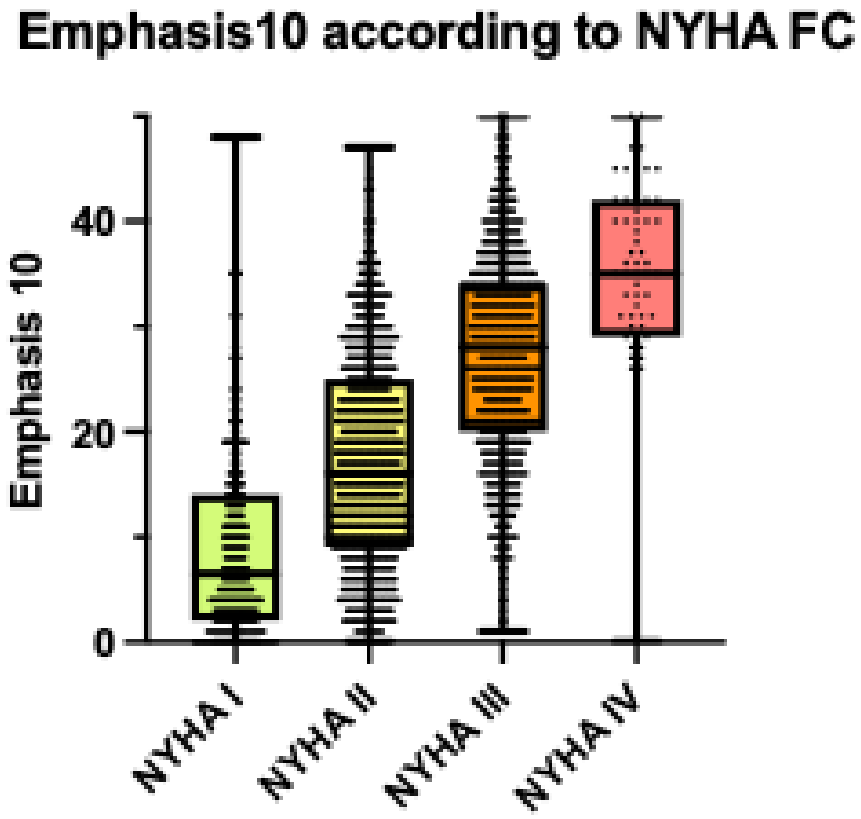
Boucly A. Data on file.

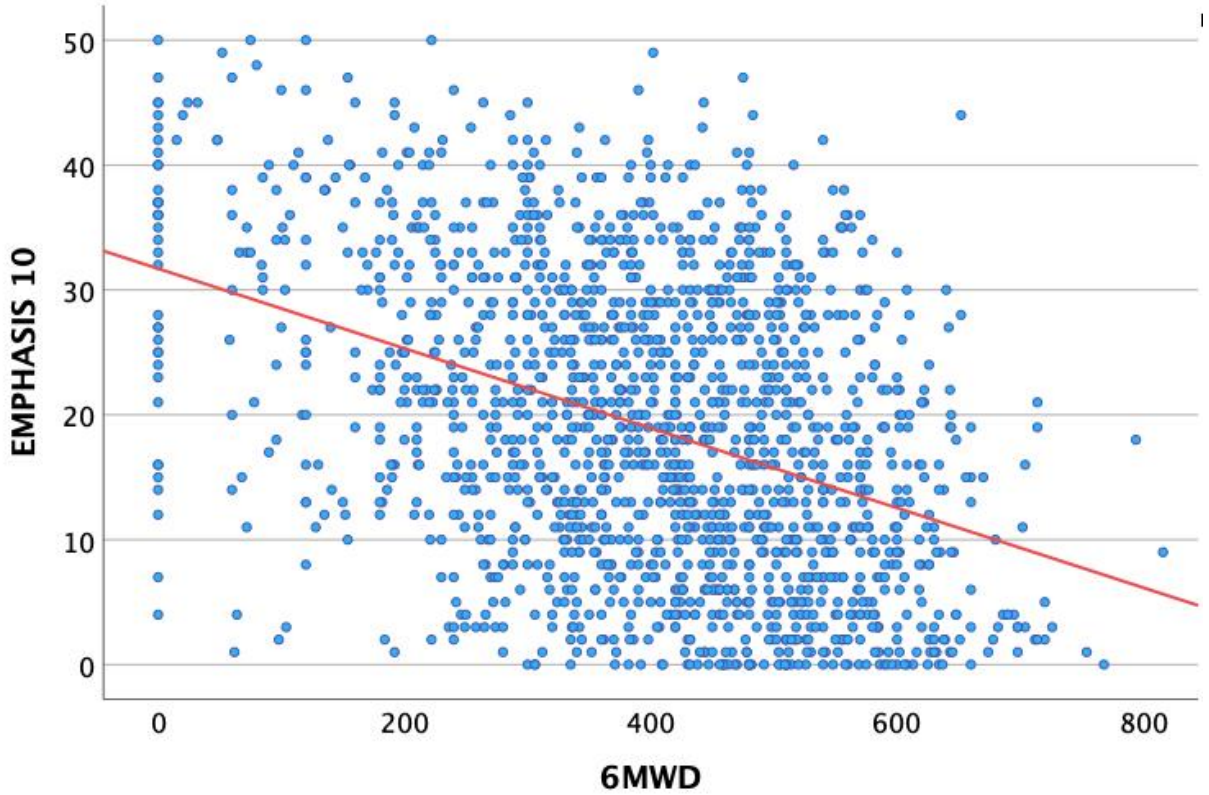
Patient characteristics (n = 2283 emPHasis10 tests)

emPHasis10 score	19 ± 12
NYHA FC, I-II / III / IV, n (%)	1483 (65) / 737 (32) / 63 (3)
6MWD, m	398 ± 150
BNP, ng/L NT-proBNP, ng/L	91 (31 – 221) 230 (82 – 748)
RAP, mmHg	7 ± 4
mPAP, mmHg	40 ± 12
PAWP, mmHg	10 ± 4
Cardiac output, L/min	5.2 ± 1.6
Cardiac index, $L/min/m^2$	2.9 ± 0.8
PVR, WU	6 ± 4
SVI, mL/m^2	38 ± 12
SvO_2, %	68 ± 7

Boucly A. Data on file.

Correlation of emPHasis10 score with main PH metrics

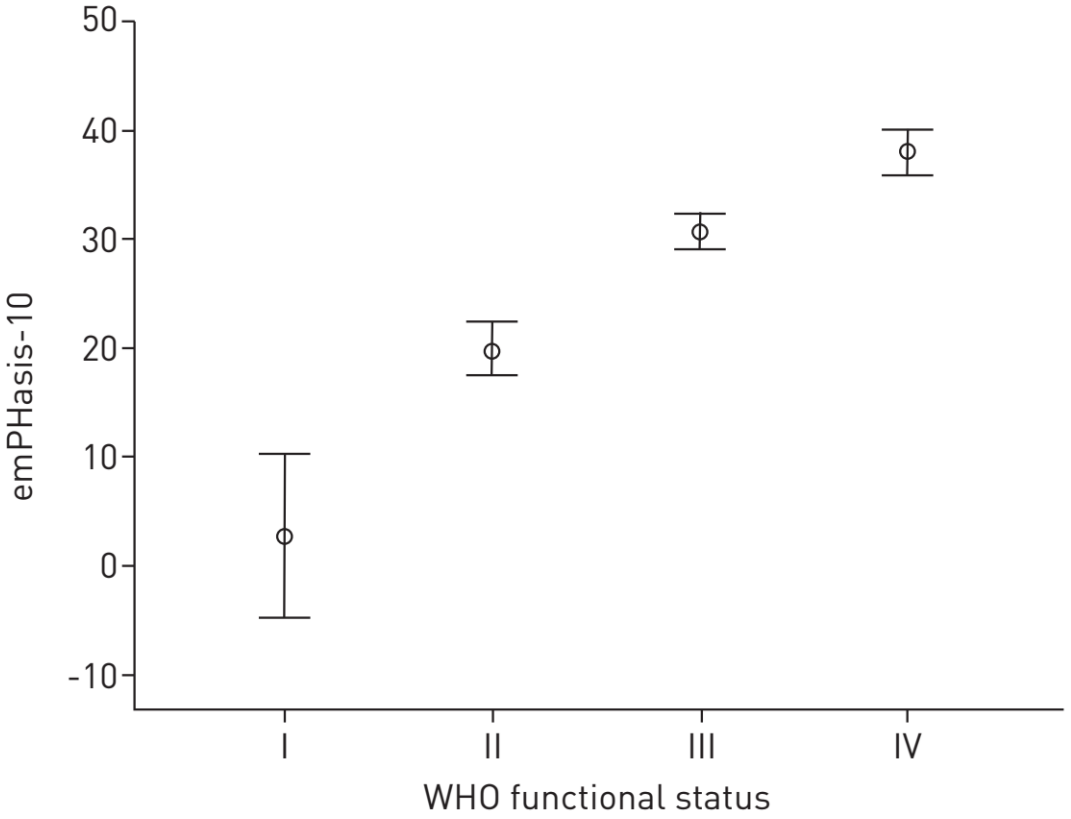


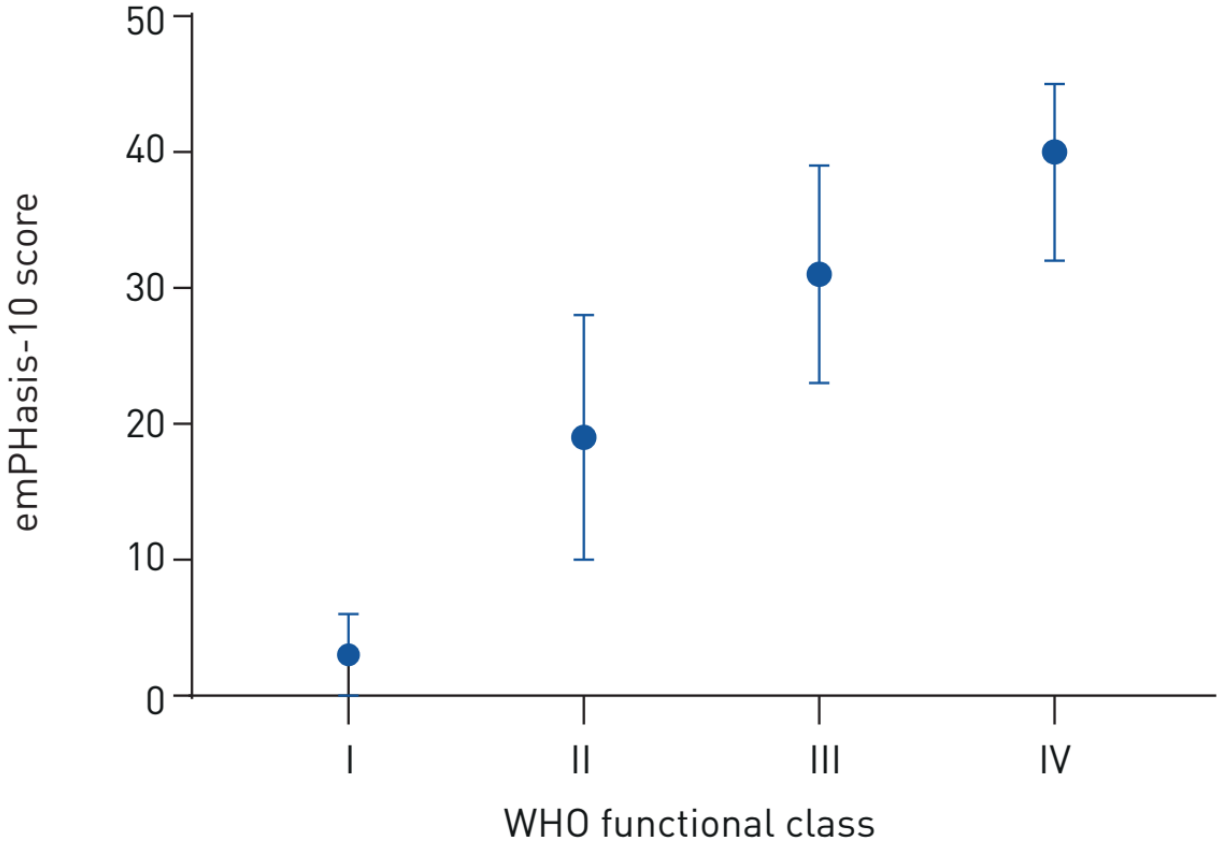


Weak correlation with pulmonary haemodynamics and NT-proBNP

Boucly A. Data on file.

Correlation of emPHasis10 score with functional class (UK)

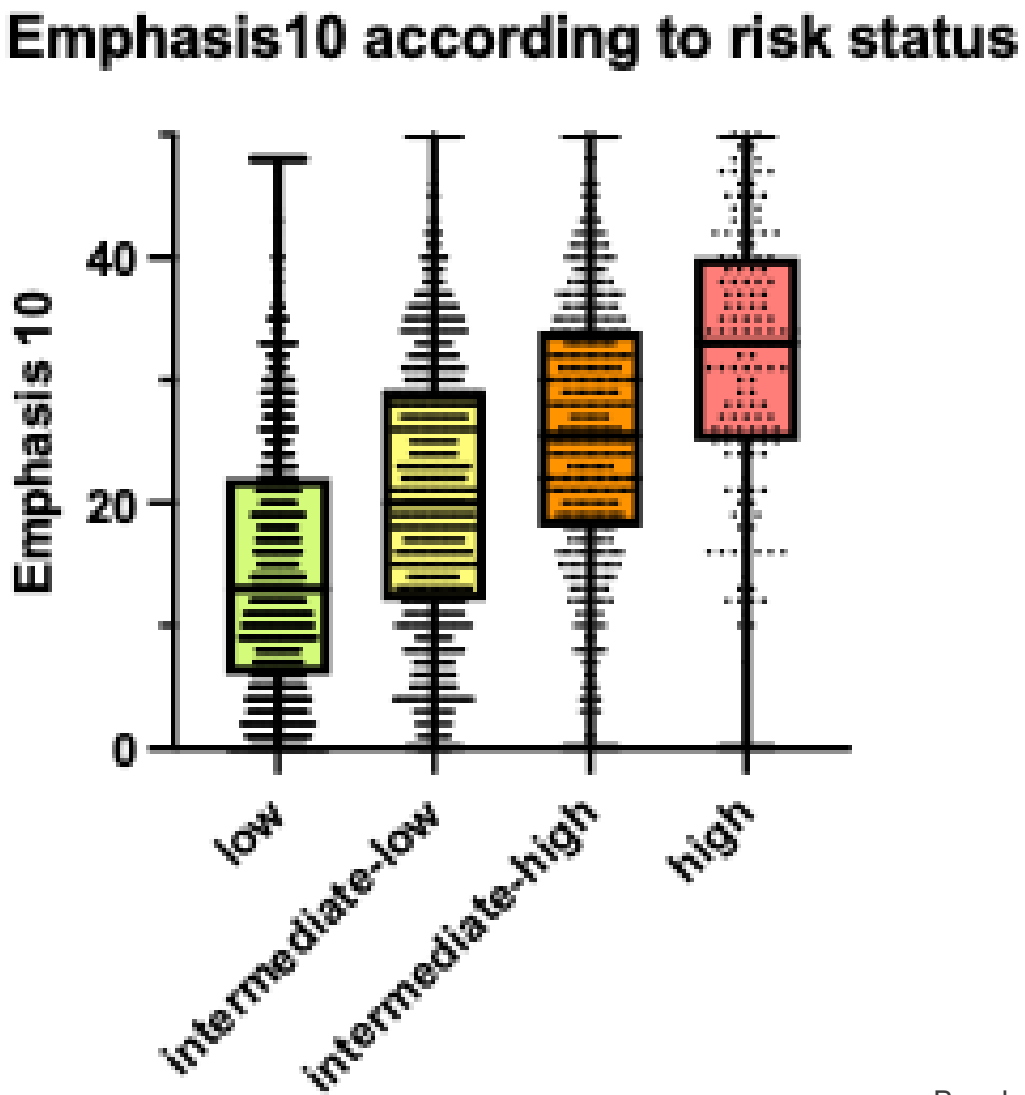




Yorke J, *et al. Eur Respir J* 2014; 43: 1106–1113; Lewis RA, *et al. Eur Respir J* 2021; 57: 2000124.

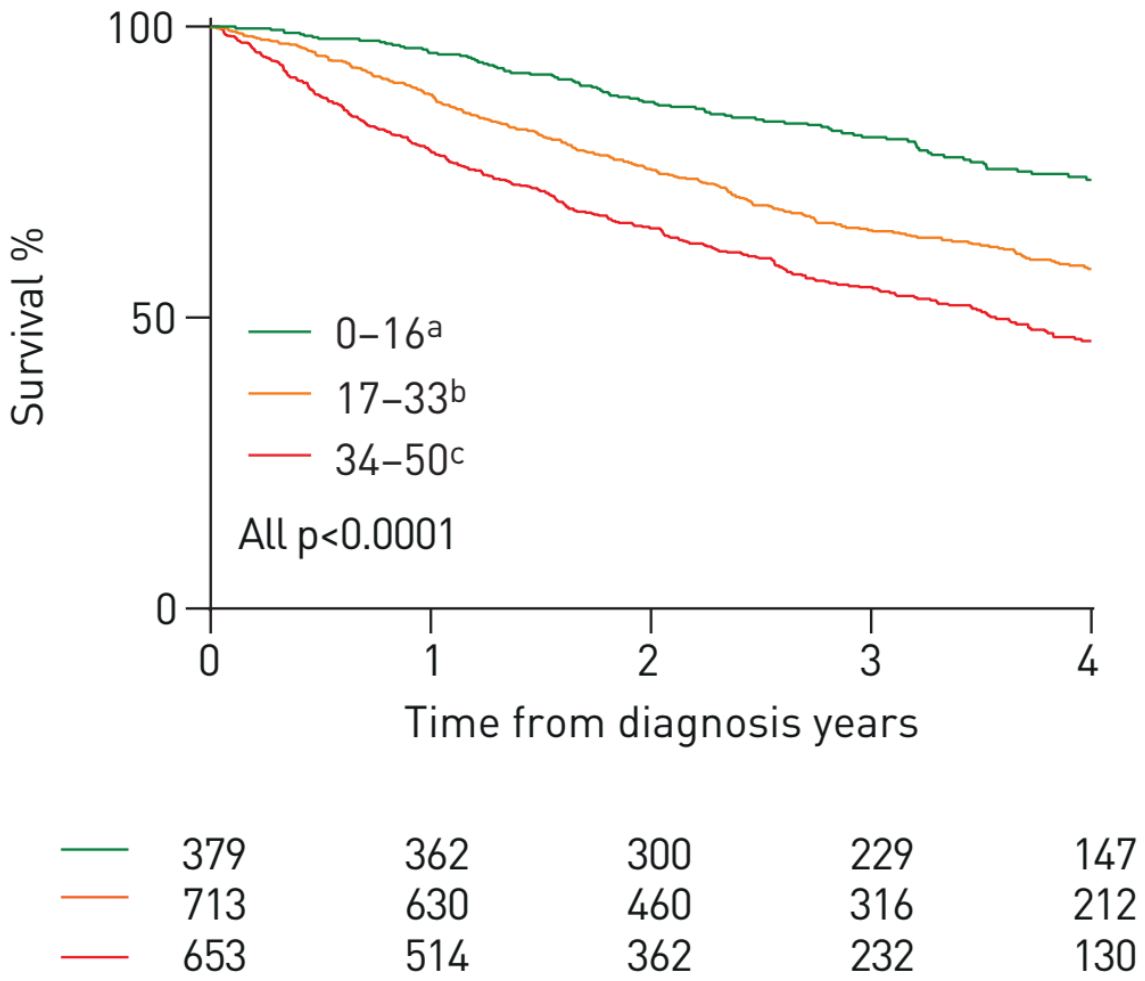
Correlation of emPHasis10 score with risk status

Risk status	Emphasis-10
Low	15 ± 11
Intermediate-low	20 ± 11
Intermediate-high	26 ± 11
High	31 ± 11



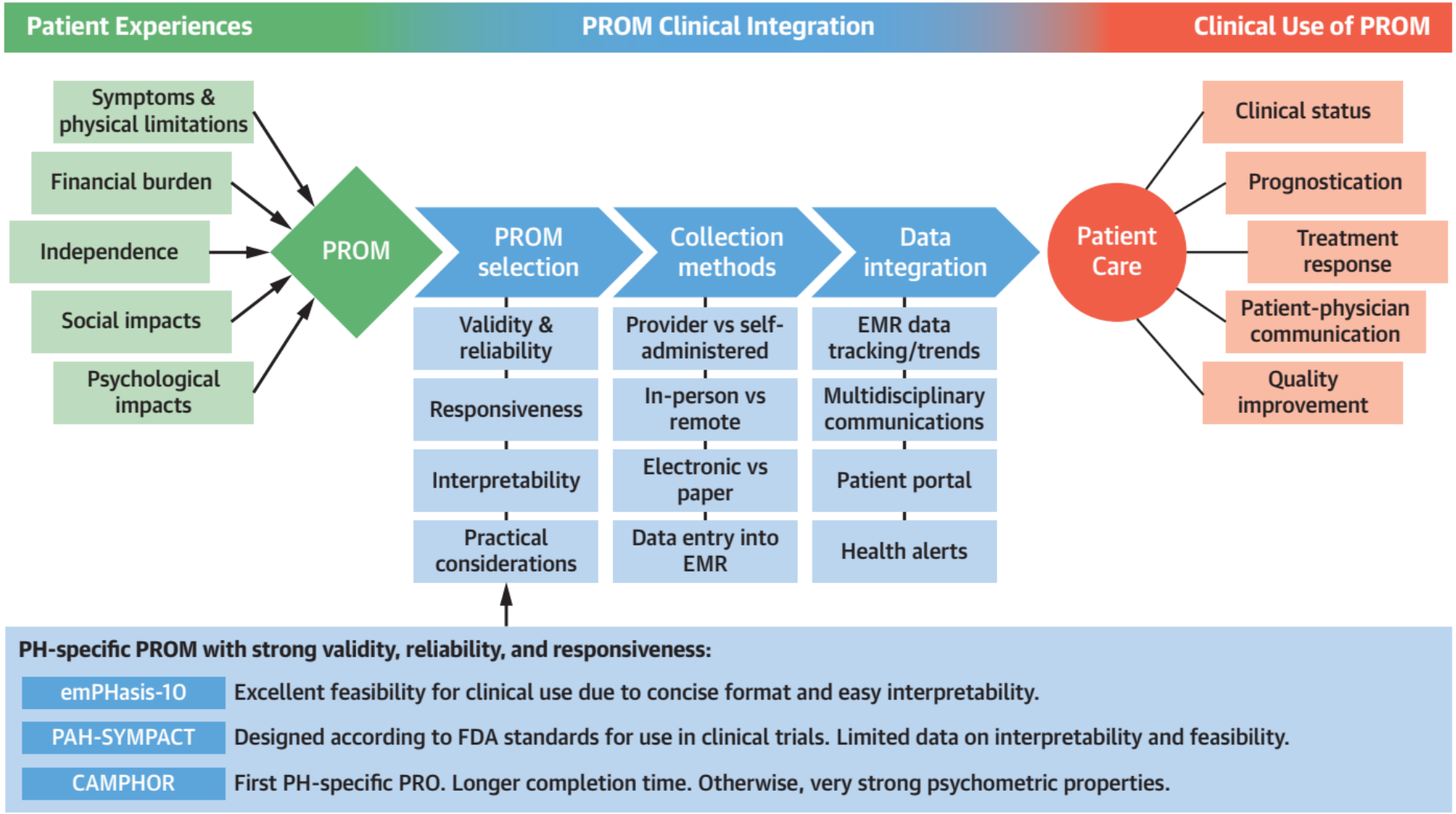
Boucly A. Data on file.

emPHasis10 score predicts outcomes (UK)



Lewis RA, *et al. Eur Respir J* 2021; 57: 2000124.

Clinical use of PROMs in Pulmonary Hypertension



Rose SW, *et al. J am Coll Cardiol.* 2024; 12: 366-376.

Thank you for your attention!